"Success is not final, failure is not fatal: it is the courage to continue that counts."

Rare Disease Advocacy World

The 8th Annual World Orphan Drug Congress was held in Maryland this past April. Rare Disease Advocacy World highlights the industry game changers that are accelerating rare disease research and drug development. It is an excellent opportunity for patient advocacy groups to meet with key stakeholders and network on topics, including global access to treatments, gene therapy development, orphan drugs policy, gene editing, partnering and investment, along with round table discussions on critical issues that center on treatment and non-treated patients. In addition, the platform includes patient advocacy group discussions.

The National MPS Society presented data on newborn screening initiatives, the relevance of patient registries, and the responsibility of today's nonprofit organizations of securing the patient voice in both of these efforts. The future landscape can be driven in part by patient advocacy groups–a collaborative voice is key to securing optimum outcomes.

It was an extraordinary few days, escalating conversations for MPS and ML and critical concerns of insurance issues, access to treatments and sharing stories for those who do not have treatment yet.

For more information about this conference, click here.

May 15 is International MPS Awareness Day!

This year we have created a calendar challenge for the first 15 days of May. The National MPS Society would like to hear from families and individuals who try to complete the tasks on the calendar each day and how you have worked to spread awareness. These daily challenges lead up to the big day, where we encourage you to wear purple, spread the word, and plant the airplane seed packets you received in the mail as we work to grow awareness.

Use #flyformps and #mpsawareness2018 on social media, and make sure to check out www.mpsday.com to fly virtual planes, learn more, and share with others!

Ultragenyx 2018 Patient Day

On April 7, National MPS Society Vice Chairman Kim Whitecotton, along with her husband, Tom, and son, Scotty (MPS II), attended the 2018 Ultragenyx Patient Day event, which honors individuals living with rare diseases.

The Whitecottons met new friends with different rare diseases, and had the opportunity to speak with Dr. Emil Kakkis, CEO of Ultragenyx.

The event included food trucks, face painting, a jumphouse, obstacle course and games. Performer Rachel Platten sang her famous "Fight Song," which was perfect for the superhero-themed event.

"It was a great opportunity to meet so many dedicated people working on rare disease treatments," said Kim. "What inspired us most was listening to the stories of three families and their incredible rare disease journey. We could see ourselves in every one of their stories. The fight for answers, and the will to never give up!"

Meet Gabriel--a happy, (almost) 3-year-old boy who has Sanfilippo syndrome! Gabe is the son of Ashton Baird and Matt Clark, and is big brother to Dalton. The family resides in Delaware, OH. The brothers are very close and play well together. Gabriel loves Mickey Mouse, playing with cars and going for walks. His mom says that, just like all children, Gabriel loves to play and learn new things.

Drake Lucas was born Jan. 25, 2018. At almost two weeks of age, his pediatrician called to notify his family that a newborn screening test showed an abnormal result. After additional testing, Drake was diagnosed with MPS I. Drake's family connected with support groups right away, and started the process of relocating their family temporarily for a transplant process. Newborn screening initiatives are crucial to having more stories like Drake's, where families have more treatment options and information available with an early diagnosis. For more about the Lucas family and their journey, visit his Courage Page.

National MPS Society Supports the RARE Act

The Rare Disease Advancement, Research and Education Act of 2018 (H.R. 5115), if enacted, could have substantial beneficial impacts on the rare disease patient community. Introduced by Congressmen Carson and Costello, this legislation will:

- Increase rare disease research funding at the National Institutes of Health by authorizing an additional $10 million each year for the Rare Disease Clinical Research Network;
- Create new efforts to track and combat rare diseases at the Centers for Disease Control and Prevention by creating the "National Rare Disease or Condition Surveillance System";
- Educate physicians on rare diseases through new programs at the Agency for Healthcare Research and Quality; and
- Commission additional research on how to better incentivize rare disease therapeutic development.

For more information, click here.

BioMarin North America Patient Advocacy Forum

Practical Digital Solutions for Community Engagement

The National MPS Society participated in the BioMarin North America Patient Advocacy Forum with more than20 additional rare disease organizations throughout the United States and Canada. This year's topic was Practical Digital Solutions for Community Engagement. The Society was asked to speak on communication support during life transitions.

The event, hosted by BioMarin, included round table discussions on all social media and website formats with patient advocacy organizations, BioMarin employees and professional communication experts. Goals of

UPCOMING EVENTS

MPS Awareness Day
May 15, 2018

Million Dollar Bike Ride
May 20, 2018

4th Annual Cooper's Troopers BBQ
June 2, 2018

First Annual Toss for Tony
June 9, 2018

2018 International Symposium
Aug. 2-4, 2018

San Diego Superhero 5K Run & Walk
Aug. 5, 2018

Jeff Bardsley MPS Golf Classic
Oct. 8, 2018

In Memoriam

Trenton Greer
(MPS IIIA)
03/12/09 – 4/12/18

Elizabeth Hulett
(MPS I)
06/17/03 – 04/23/18

the event were to determine best practices for rare disease organizations and offered opportunities throughout the day to learn from one another. BioMarin also included The Havas Team, a professional company with decades of experience in marketing and communications.

The event included a visit to the San Rafael campus where organizations were presented with a "lock of love" to secure on the artistic designed trees. Although Society representatives were unable to attend, we are hoping to visit the campus in the future and secure our MPS Society lock!
Thanks to BioMarin for these incredible opportunities to network and gain valuable knowledge with the most inspiring organizations in North America.

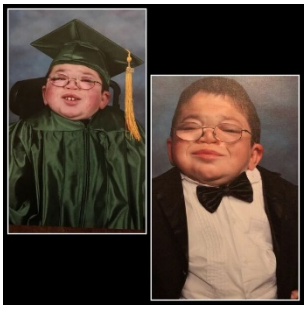
(Pictured: Terri Klein and Sharon King.)

Industry Updates
MPS II (Hunter Syndrome)
Regenxbio
Regenxbio recently received the FDA Fast Track Designation for RGX-121 Gene Therapy for the Treatment of MPS II. Regenxbio is using its proprietary NAV Technology Platform designed to deliver the human iduronate 2-sulfatase gene directly to the central nervous system using the NAV AAV9 vector. Regenxbio will begin development of RGX-121 and begin the phase I/II trial in the coming months. RGX-121 will be evaluated in the phase I/II multi-center, open-label, multiple-cohort dose-escalation study in children with MPS II. For more information on the clinical trial and design eligibility criteria, click here.

Sangamo Therapeutics
In April, Sangamo released preclinical murine study data, which provided proof of concept for the development of SB-913, an in vivo genome editing product candidate. Male MPS II model mice, between six and nine weeks of age, were injected with one of three increasing dose levels of a genome editing treatment consisting of AAV2/8 vectors encoding a pair of ZFNs and a

Maci Eickman
(MPS I)
10/1/15 – 04/30/18

Sloane Skelton
(MPS I)
12/18/16 – 05/01/18



Austin Marine received a scholarship to help him attend Alamance Community College through a specialized program. Though he has a diagnosis of ML II/III, Austin finds it important to go to college to make friends and continue his education. Austin's favorite thing about going to school is getting to talk to people, which makes him feel happy. Congratulations, Austin!

corrective human IDS gene. The data resulted in greater than 95% reduction in GAG substrate levels. In addition, it was reported that at the highest dose level, there was prevention of the development of neurocognitive deficit in the mice.

Currently the ongoing CHAMPIONS Study, a phase I/II clinical trial assessing the potential safety and efficacy of SB-91, will include up to nine adult males with attenuated MPS II. This treatment uses Sangamo's SFN genome editing technology delivered intravenously via AAV6 vectors and is a single-treatment strategy intended to provide stable, continuous production of the IDS enzyme. The information was published in the April 2018 issue of *Molecular Therapy*. Read more.

MPS IIIA (Sanfilippo Syndrome)

Abeona

In late April, Abeona announced that the FDA granted the first Regenerative Medicine Advanced Therapy (RMAT) Designation to ABO-102 Gene Therapy in MPS IIIA. RMAT was established under the 21st Century Cures Act and is an expedited program for the advancement and approval of development of Abeona's ABO-102.

According to the 21 Century Cures Act, a regenerative medicine is eligible for the designation if it is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition, and clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition.

Working more closely with the FDA will elevate the collaboration and thorough communicative pathways between industry and regulations. Read more.

Lysogene

Lysogene is developing a gene therapy candidate for the treatment of MPS IIIA, which is designed to use the AAVrh10 vector to deliver the human SGSH gene directly to the central nervous system. The company expects to begin enrollment in a pivotal phase II/III clinical trial in the second half of 2018. The company recently completed the enrollment for the first multi-national observational study in MPS IIIA which will function as the non-concurrent control for the first pivotal trial for MPS IIIA in 2018. Lysogene has obtained orphan drug designation from the

Welcome MPS Society new members:

Mariel Abreu, New York, adult with MPS VI

Natalie Anaya Luna and Bruno Marie Bordes, Puerto Rico, parents of Juliette Marie Anaya, MPS I

Allicia and Randall Gibbs, Kansas, parents of Jamea Gibbs, MPS IIIA

Jenny and Travis Greer, Florida, parents of Trenton Greer, MPS IIIA

Katie Miner, Ohio, mother of Kiera Miner, MPS VI

Alex and DeAnna Timbrook, Texas, parents of Savannah Timbrook, MPS VI

Mariah Williams, Texas, mother of Knoah Williams, MPS I

Katie Willmann, Missouri, mother of Antonio "Tony" Willmann, MPS II

Save the Date!

San Diego Superhero 5K & Memorial Walk

The National MPS Society will host a chip-timed 5K run and one-mile memorial walk at 8:00 a.m. PDT on Sunday, Aug. 5. Please join in celebrating our super heroes as we conclude the 15th

EMA and FDA and rare pediatric designation by the FDA. Read more.

MPS Race for a Cure

The National MPS Society hosted its third annual MPS Race for a Cure 5K Run and One Mile Memorial Walk on Sunday, April 29, in Napa, CA. The race was extremely well attended with more than 600 participants! Our families and friends had a great time raising funds for the Society and catching up while enjoying the beautiful grounds of the Veterans Home of California.

This event continues to be a success year after year due to the generous time and support our race committee members provide. This year was no exception; families and local businesses organized an outstanding auction and raised more than $60,000! A nice time was had by all with entertainment for the kids, live music and a post-run lunch.

International Symposium on MPS and Related Diseases in San Diego. The race will take place at the lovely Spanish Landing Park, which is just a short walk from the Sheraton Hotel on Harbor Island Drive. Advance registration coming soon!

The Amazing Charitable IRA Rollover Gift

If you are at least 70 1/2 years old, you can make a direct charitable gift to the National MPS Society of up to $100,000 in a single year from your IRA account without having to pay federal tax on the withdrawal. This permanent American tax provision was preserved in the Tax Cuts and Jobs Act of 2017. Such a gift will qualify for your “required minimum distribution.”

Read more.

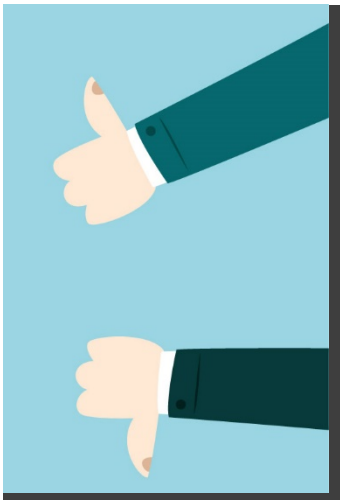
More than 100 representatives from pharmaceutical industry sponsors made it a priority to attend the event this year, and we are inspired by their dedication to the fight against MPS and ML. Because of their support, we have been able to consistently raise tens of thousands of dollars each year to fund research and family support programs.

MILLION DOLLAR BIKE RIDE

RAISING MONEY FOR RARE DISEASE RESEARCH

Sunday, May 20
7:30 a.m.
Highline Park
21st and Chestnut St.
Philadelphia, PA

It's not too late to support our team as we reach for our fundraising goal of $100,000! Click here for more information.



We want to hear from you!
Please let us know what stories and information you'd like to see in future editions of Courage. Click here to send story ideas or submissions.

20th Annual Klenke Bowl

The 20th Annual Klenke Bowl was held April 7 at Poplar Junction Lanes & Lounge in Highland, IL, in loving memory of Kraig Klenke (MPS II). The event was hosted by Kris Klenke and family, and included a bowling competition, silent auction, bake sale, and raffle. Five MPS families were in attendance. The Klenke family would like to express their gratitude for the outpouring of support from the community over the past 20 years.